CRITICAL PATH INSTITUTE






Critical Path Institute Annual Report FY22

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Our Mission

Critical Path Institute is a catalyst for innovation
that accelerates the path to a healthier world

Our Commitment

C-Path is committed to fostering open, precompetitive collaboration between multiple stakeholders, which leads to innovation and acceleration of medical product development.

Collaboration

By bringing together industry leaders, regulators, scientists, academic researchers, patients, clinicians and patient groups who share a common goal, C-Path generates streamlined, therapeutic solutions for those with acute needs.

Acceleration

C-Path's ability to generate and deliver solutions which streamline the development process helps to alleviate lengthy bottlenecks and information silos. Stakeholders are able to meet key development milestones in their pursuit of global health and well-being.

Innovation

Since its inception in 2005, C-Path's partnerships have delivered actionable solutions to very difficult healthcare obstacles, including, but not limited to, first-in-class regulatory endorsed tools and solutions, with **impact** such as:

A translational *in vitro* platform, and a physiologically based lung model that contributed to the first new treatment and regimen for tuberculosis in more than 50 years

A first-in-class imaging biomarker for polycystic kidney disease, which contributed to the first-ever drug to be approved to slow the progression of the disease

The first-ever computerized tool endorsed by the U.S. Food and Drug Administration and the European Medicines Agency to transform clinical trial design in Alzheimer's disease

Clinical outcome assessments that have optimized incorporation of the patient's voice in the evaluation of treatment efficacy for asthma, depression, irritable bowel syndrome, myelofibrosis and non-small cell lung cancer

Novel approaches to research that have led to the regulatory endorsement of viable biomarkers in Alzheimer's and Parkinson's diseases, kidney disease, type 1 diabetes and kidney transplantation

Dear Friends and C-Path Supporters,

As the newly appointed CEO of C-Path, I am humbled to have joined an organization that continues to forge an inspiring vision for its growth and impact, and I embrace the profound responsibility of ensuring C-Path's long-term success by building upon our shared goals.

Thanks to the scientists, academic organizations, companies, as well as regulatory and research agencies that form C-Path's public-private partnerships and consortia, 2022 was another banner year of innovation, transformative solutions for medical product development, and sustained growth for C-Path's world-class initiatives and collaborations.

This is an incredibly exciting time to be part of the medical product development sector. Advances in medical technology and data-sharing are giving us tools that only years ago seemed far out of reach. As the leading nonprofit organization dedicated to improving and streamlining the process of medical product development, we will continue to invest in our strategic expansion and leverage shared expertise to develop solutions for some of the most demanding drug development puzzles existing today.

Daniel M. Jorgensen, MD, MPH, MBA
CEO, C-Path

At C-Path, we're committed to bringing together the best minds to tackle the biggest issues. So, we understand that we can't do it alone. Only with the support of our partners and donors, the backing of foundations and regulatory agency advisors, collaborations with biotech and pharmaceutical companies, other key industry stakeholders and the strength of patient groups, will we be able to continue building upon our successes and advance bold new breakthroughs and life-altering innovations.

Throughout the following pages, you will find the story of a nonprofit organization that is working to transform the world of medical product development and, if I may be so bold, is delivering on its promise to work tirelessly to accelerate the development of new therapies.

I would like to thank all of you — our staff, our partners, collaborators and supporters around the world. We share in our successes, and we are buttressed by our shared commitment.

With gratitude and best wishes,

FY22 Year in Review

C-Path's 17th year in operation brought about its biggest year yet in funding and growth. Active programs and consortia increased to nearly 25, with more collaborators, members and member organizations dedicated to achieving tangible, actionable results by fostering collaboration between industry executives and scientists, academic researchers, regulators and patient groups.

A snapshot of our growth this year, includes:

Launch of Pioneering Translational Therapeutics Accelerator

With an award from the Frederick Gardner Cottrell Foundation, a nonprofit organization established by Research Corporation Technologies, Inc., C-Path launched a first of its kind program for the Institute called the Translational Therapeutics Accelerator. TRxA is a unique, global effort focused on supporting academic scientists in defining optimal strategies for advancing new, cutting-edge therapeutics from the lab to patients.

First EMA Qualification Opinion of Biomarkers for T1D Prevention Trials

In April 2022, C-Path's Type 1 Diabetes Consortium was issued a positive qualification opinion for pancreatic islet autoantibodies as enrichment biomarkers for type 1 diabetes prevention trials from the EMA. The model-based qualification will make tools to assist in the identification and selection of patients with a likelihood of progressing to a T1D clinical diagnosis, in trials of reasonable duration, publicly available.

John Crawford,
Parkinson's disease patient

Opening of C-Path Nonprofit European Office in Amsterdam

In support of C-Path's mission to catalyze innovation that accelerates the path to a healthier world, the nonprofit announced the opening of its European office in the Netherlands, which focuses on the strengthening of existing, and development of future, activities in Europe. This will facilitate C-Path's advancement to develop novel regulatory-grade methodologies to accelerate medical product development around the globe.

Fastest and Biggest Patient-Level Data Integration at C-Path, Benefiting Neonates

As a part of an FDA-sponsored project to generate actionable real-world evidence from real-world patient-level data for neonatal drug development, the International Neonatal Consortium and Tufts Medical Center came together to transfer electronic health record data, marking the first time C-Path integrated this type of information. Additionally, INC also received fully anonymized electronic patient record data from the National Neonatal Research Database as part of efforts to accelerate medical product development for a chronic lung disease that affects premature infants. It was C-Path's largest data transfer to date. This was the first time C-Path received electronic health record data from the United Kingdom and included data from a network of nearly 200 hospitals. Then in December 2021, the Consortium embarked on a seminal data-sharing collaboration with the Eunice Kennedy Shriver National Institute of Child Health and Human Development of the National Institutes of Health.

Expansion of C-Path and EJP RD Global Impact and Partnership

C-Path and the European Joint Programme on Rare Diseases, an initiative that has received funding from the European Union's Horizon 2020 research and innovation program, announced a collaboration to advance technologies and methodologies that are fit for regulatory purposes to further global rare disease research and drug development.

Launch of Consortium Centered Around Patient-Focused Drug Development in Rare Diseases

In January, C-Path launched the Rare Disease Clinical Outcome Assessment Consortium, a public-private partnership focused on optimizing COA selection during medical product development for rare diseases. RD-COAC is a pre-competitive collaborative effort among C-Path and FDA, as well as the National Organization for Rare Disorders, other government agencies, and key partners in the biopharma, clinical research and patient communities that are seeking and/or developing treatments for rare diseases.

Partnership to Advance Trial Execution, Between C-Path's CDRC and ACT@POC

C-Path's CURE Drug Repurposing Collaboratory partnered with Advancing Clinical Trials at the Point of Care (ACT@POC) Coalition to assist in identifying and improving trial site resources that support trial execution. The Collaboratory was chosen to help implement digital tools that

enable more automated and straightforward data collection, consent and enrollment and adaptable common data models to collect data from electronic medical record platforms and other data sources.

Opening for Access of Unique Data and Analytics Platform for Rare Diseases, RDCA-DAP®

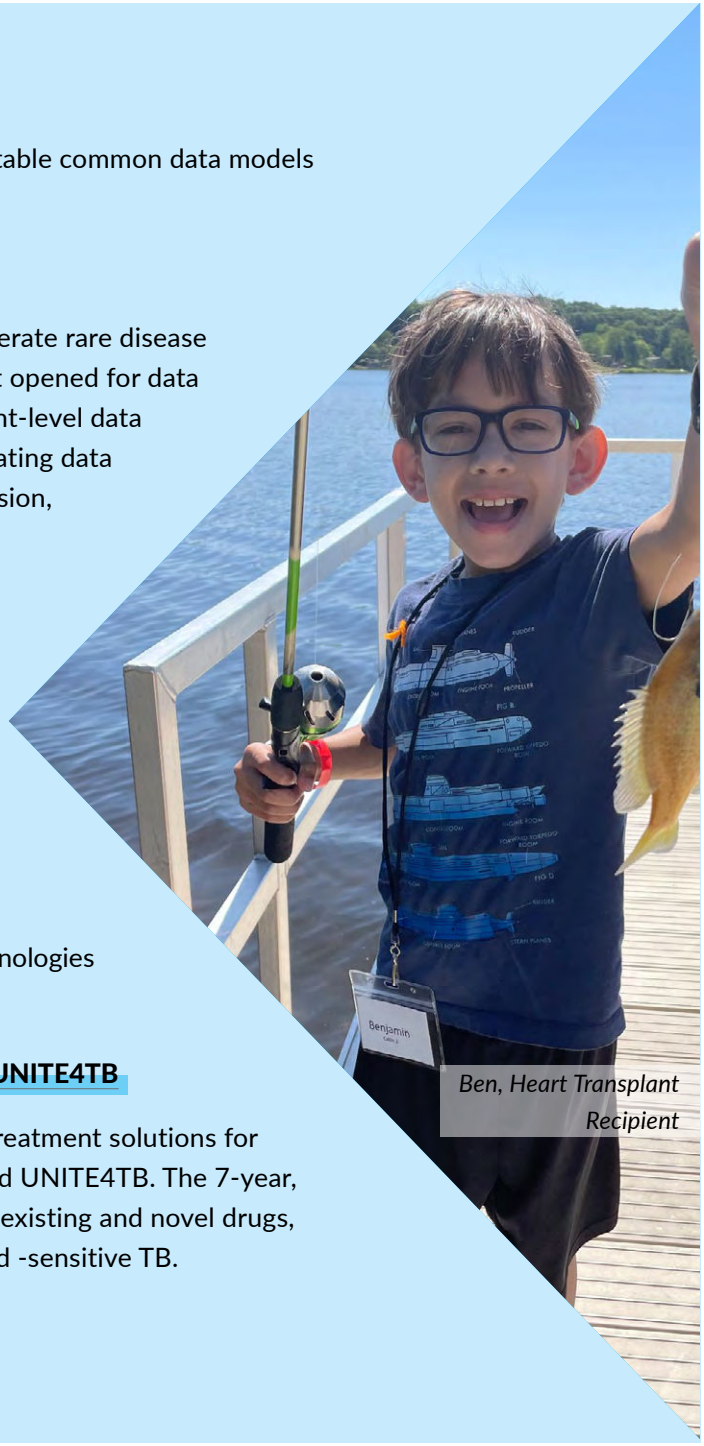
The Rare Disease Cures Accelerator-Data and Analytics Platform, the leading platform to accelerate rare disease treatment innovation, reached a significant development milestone in September 2021 when it opened for data access requests, since the start of the effort in fall 2019. The platform houses integrated patient-level data across a multitude of rare diseases contributed from organizations around the world. By integrating data in a format suitable for analytics, RDCA-DAP accelerates the understanding of disease progression, clinical outcome measures and biomarkers, and facilitates the development of mathematical models of disease and innovative clinical trial designs.

Digital Health Technology effort at C-Path Holds Key to New Parkinson's Treatments

The Digital Drug Development Tool effort of C-Path's Critical Path for Parkinson's Consortium continued to advance the understanding of how to optimally deploy digital health technologies in clinical trials for Parkinson's. This included significant progress in the understanding of key attributes of algorithms to process the data from these technologies into interpretable and actionable results, as well as a seminal publication titled "Digital Progression Biomarkers as Novel Endpoints in Clinical Trials: A Multistakeholder Perspective," co-authored by C-Path expert, Diane Stephenson, Ph.D. The paper highlighted how new remote monitoring technologies present a tremendous opportunity to advance digital medicine.

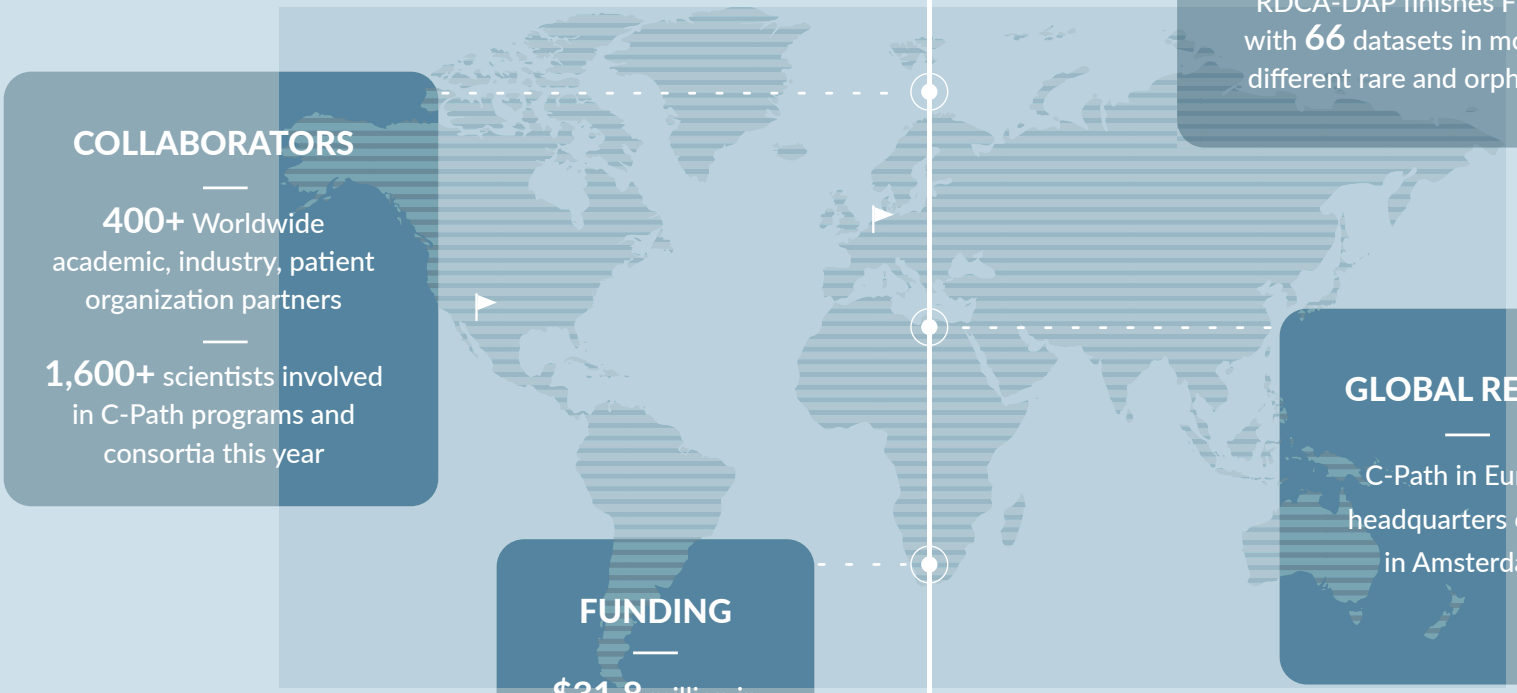
New Era in Tuberculosis Treatment Development Marked by Partnership between C-Path and UNITE4TB

To advance anti-tuberculosis science and enable the progression of new, safe, and affordable treatment solutions for TB patients worldwide, C-Path joined a new consortium of 30 partners from 13 countries called UNITE4TB. The 7-year, €185 million project, aims to accelerate and improve the clinical evaluation of combinations of existing and novel drugs, with the goal of developing new and highly active TB treatment regimens for drug-resistant and -sensitive TB.



Ben, Heart Transplant Recipient

Our Best Year Yet...



DATA INTEGRATION

More than **100%** growth in individual subject data, through a **20%** increase in new datasets integrated by C-Path's Data Collaboration Center

RDCA-DAP finishes FY22 strong with **66** datasets in more than **24** different rare and orphan diseases

COLLABORATORS

400+ Worldwide academic, industry, patient organization partners

1,600+ scientists involved in C-Path programs and consortia this year

GLOBAL REACH

C-Path in Europe headquarters opens in Amsterdam

FUNDING

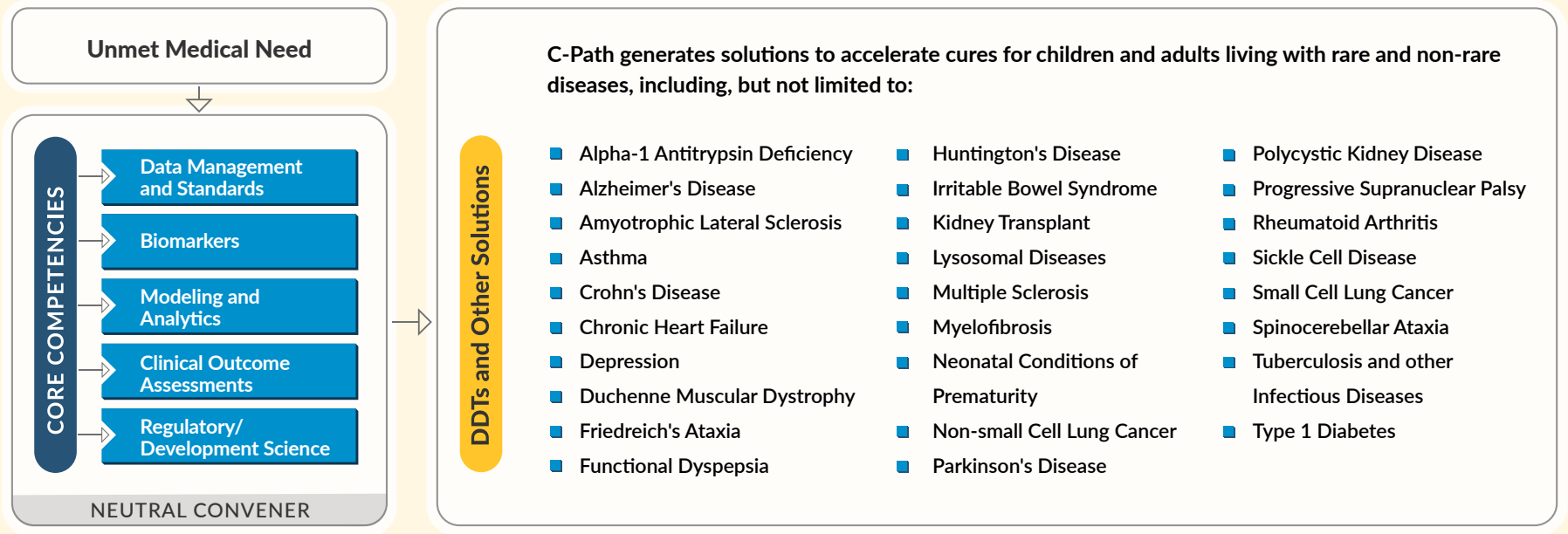
$31.8 million in funding, **+28%**

Core Competencies

The sharing of information and data under a neutral environment serves as the foundation for C-Path to spearhead actionable solutions that address specific unmet needs in the drug development process. Since 2005, the Institute has broadened its role and excelled in five core areas:

1. Data Management and Standards
2. Biomarkers
3. Modeling and Analytics
4. Clinical Outcome Assessments
5. Regulatory Science

These Core Competencies are the foundation for C-Path to generate solutions that de-risk decision making in the development and regulatory review process, primarily focused in the areas of neuroscience, immunology and inflammation, infectious diseases, safety science, pediatrics and rare and orphan diseases.



Financials

ASSETS

Cash and Cash Equivalents	$	16,868,064
Certificates of Deposit	$	—
Accounts Receivable	$	3,141,154
Property and Equipment, Net	$	72,592
Other	$	59,762
Total Assets	**$**	**20,141,572**

LIABILITIES AND NET ASSETS

LIABILITIES

Accounts Payable	$	1,073,348
Accrued Expenses	$	906,082
Deferred Revenue*	$	6,055,129
Deferred Rent	$	82,473
Total Liabilities	**$**	**8,117,032**

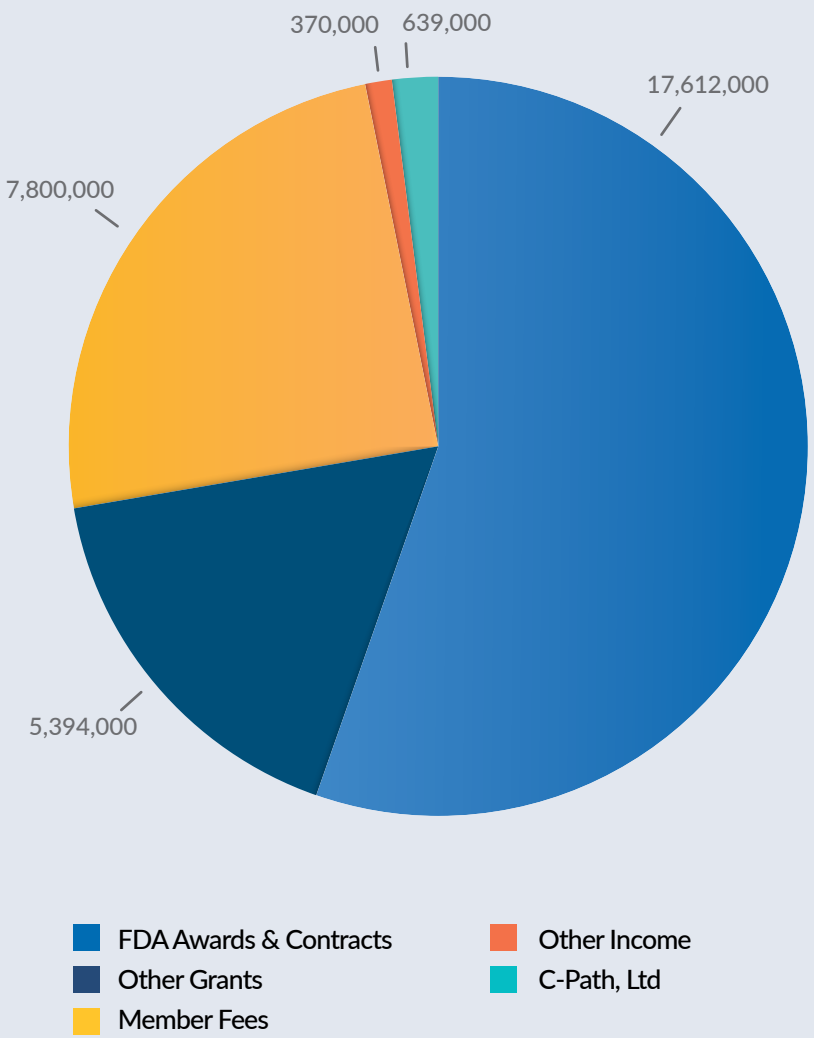
NET ASSETS

Undesignated	$	2,483,489
Board Designated**	$	3,128,493
Coordinating committee Designated	$	3,661,072
Property and Equipment	$	72,592
Donor Restricted	$	2,678,894
Total Net Assets	**$**	12,024,540
Total Liabilities and Net Assets	**$**	**20,141,572**

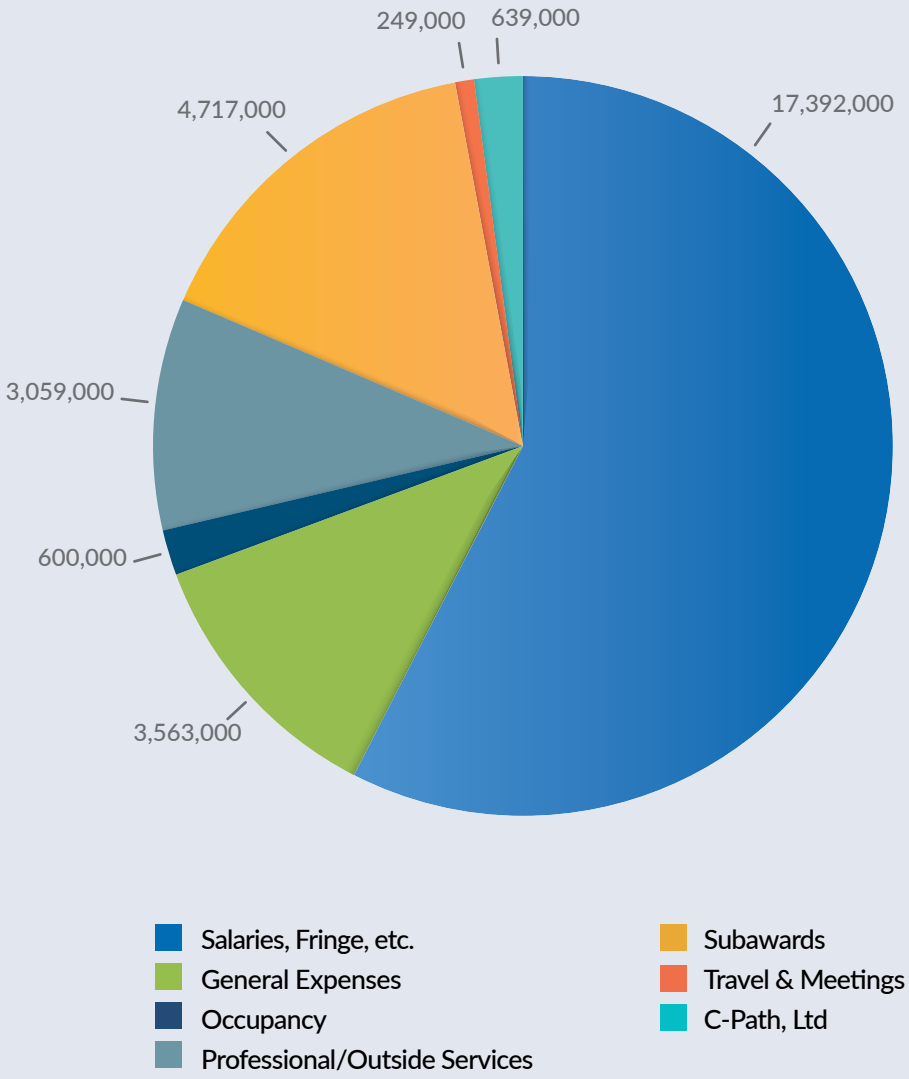
* Pre-awarded funds received for grants and consortia

** Consortia fees managed by C-Path to support consortia activities

C-PATH 2022 FISCAL YEAR REVENUE: $ 31,815,000



C-PATH 2022 FISCAL YEAR EXPENSES: $ 30,219,000



Our Collaborators

Scientists

By addressing broad process inefficiencies, C-Path enables industry and academic scientists to focus on their true goal: developing therapies and medical products that will improve human health and well-being.

Regulatory Agencies

Regulatory agencies play a critical role as stewards of public health, and regulators share valuable non-competitive insights at every stage of the C-Path process.

Patients and Patient Advocacy Groups

C-Path relies on insights from patients living with diseases, their caregivers and advocacy groups that support them around the world, to help make the process of developing cures, therapies, and medical products more efficient.

Medical Product Development Executives

By increasing process efficiencies, C-Path helps industry stakeholders focus on their organizations' goals and meet their investor milestones.

Donors and the Philanthropic Community

C-Path's work to accelerate the development of products and therapies that can benefit patients whose conditions lack safe and effective treatments would not be possible without the support from donors and foundations.

For a full list of our collaborators, visit c-path.org/c-path-collaborators.

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Looking Ahead

From C-Path's humble beginnings, it has been our mission to help forge a path to a healthier world. We have overcome many challenges, but throughout our history these obstacles have been met head-on and we have continued to deliver cutting-edge solutions and advancements. Today, C-Path is known as a global force for problem-solving and effective collaboration.

We owe an immeasurable debt to our supporters around the world who have believed in our vision and contributed to our success. With every new advancement, and with every patient who has benefitted from our shared commitment, we grow more and more determined to continue pioneering new and bold directives. We look forward to continuing this journey together.

CRITICAL PATH INSTITUTE

c-path.org

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